

Title (en)
FEEDBACK ENABLED SYNTHETIC GENES, TARGET SEED MATCH CASSETTES, AND THEIR USES

Title (de)
FEEDBACK-BEREITE SYNTHETISCHE GENE, ZIELSAMENZUORDNUNGSKASSETTEN UND DEREN VERWENDUNGEN

Title (fr)
GÈNES SYNTHÉTIQUES ACTIVÉS PAR RÉTROACTION, CASSETTES CIBLES D'APPARIEMENT DE GERMES, ET LEURS UTILISATIONS

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Abstract (en)
[origin: WO2020047234A1] This invention relates to feedback-enabled synthetic genes, polynucleotide target cassettes, vectors, and pharmaceutical compositions for the purpose of providing transgene expression in target tissues that is capable of endogenous regulation for treating disorders such as dose-sensitive intellectual ability disorders, as well as methods of making and methods of using the same.

IPC 8 full level
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Citation (search report)
• [X] GADALLA KAMAL K E ET AL: "Supplemental Information Development of a Novel AAV Gene Therapy Cassette with Improved Safety Features and Efficacy in a Mouse Model of Rett Syndrome", 5 June 2017 (2017-06-05), XP055917808, Retrieved from the Internet <URL:https://ars.els-cdn.com/content/image/1-s2.0-S2329050117300621-mmc1.pdf>
• [T] SINNETT SARAH E ET AL: "Engineered microRNA-based regulatory element permits safe high-dose mini MECP2 gene therapy in Rett mice", BRAIN, vol. 144, no. 10, 29 November 2021 (2021-11-29), GB, pages 3005 - 3019, XP055918112, ISSN: 0006-8950, DOI: 10.1093/brain/awab182
• See also references of WO 2020047234A1

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